

# B:OMARIN<sup>®</sup>

## MPS IVA Selected Publications

### Open Access

Disease & Diagnosis	Harmatz P, Mengel KE, Giugliani R, et al. The Morquio A Clinical Assessment Program: baseline results illustrating progressive, multisystemic clinical impairments in Morquio A subjects. <i>Mol Genet Metab.</i> 2013;109(1):54-61. DOI: <a href="https://doi.org/10.1016/j.ymgme.2013.01.021">10.1016/j.ymgme.2013.01.021</a>
	Harmatz P, Mengel KE, Giugliani R, et al. Longitudinal analysis of endurance and respiratory function from a natural history study of Morquio A syndrome. <i>Mol Genet Metab.</i> 2015;114(2):186-194. DOI: <a href="https://doi.org/10.1016/j.ymgme.2014.10.015">10.1016/j.ymgme.2014.10.015</a>
	Wood TC, Harvey K, Beck M, et al. Diagnosing mucopolysaccharidosis IVA. <i>J Inherit Metab Dis.</i> 2013;36(2):293-307. DOI: <a href="https://doi.org/10.1007/s10545-013-9587-1">10.1007/s10545-013-9587-1</a>
Elosulfase alfa	Hendriksz CJ, Burton B, Fleming TR, et al. Efficacy and safety of enzyme replacement therapy with BMN 110 (elosulfase alfa) for Morquio A syndrome (mucopolysaccharidosis IVA): A phase 3 randomised placebo-controlled study. <i>J Inherit Metab Dis.</i> 2014;37(6):979-990. DOI: <a href="https://doi.org/10.1007/s10545-014-9715-6">10.1007/s10545-014-9715-6</a>
	Hendriksz CJ, Parini R, AlSayed MD, et al. Long-term endurance and safety of elosulfase alfa enzyme replacement therapy in patients with Morquio A syndrome. <i>Mol Genet Metab.</i> 2016;119(1-2):131-143. DOI: <a href="https://doi.org/10.1016/j.ymgme.2016.05.018">10.1016/j.ymgme.2016.05.018</a>
	Hendriksz CJ, Giugliani R, Harmatz P, et al. Multi-domain impact of elosulfase alfa in Morquio A syndrome in the pivotal phase III trial. <i>Mol Genet Metab.</i> 2015;114(2):178-185. DOI: <a href="https://doi.org/10.1016/j.ymgme.2014.08.012">10.1016/j.ymgme.2014.08.012</a>
	Cleary M, Davison J, Gould R, et al. Impact of long-term elosulfase alfa treatment on clinical and patient-reported outcomes in patients with mucopolysaccharidosis type IVA: results from a Managed Access Agreement in England. <i>Orphanet J Rare Dis.</i> 2021;16:38. DOI: <a href="https://doi.org/10.1186/s13023-021-01675-x">10.1186/s13023-021-01675-x</a>
	Mitchell JJ, Burton BK, Bober MB, et al. Findings from the Morquio A Registry Study (MARS) after 6 years: long-term outcomes of MPS IVA patients treated with elosulfase alfa. <i>Mol Genet Metab.</i> 2022;137:164-172. DOI: <a href="https://doi.org/10.1016/j.ymgme.2022.08.007">10.1016/j.ymgme.2022.08.007</a>
Management Guidelines	Akyol MU, Alden TD, Amartino H, et al. Recommendations for the management of MPS IVA: Systematic evidence- and consensus-based guidance. <i>Orphanet J Rare Dis.</i> 2019;14(1):137. DOI: <a href="https://doi.org/10.1186/s13023-019-1074-9">10.1186/s13023-019-1074-9</a>

If you would like a copy of any publication, please contact us at [medinfo@bmrn.com](mailto:medinfo@bmrn.com) or through our [web request form](#).